Axatilimab-csfr (NIKTIMVO)
National Drug Monograph
March 2026

VA Pharmacy Benefits Management Services and National Formulary Committee

The purpose of VA National Formulary Committee drug monographs is to provide a focused drug review for making formulary decisions. The Product Information or other resources should be consulted for detailed and most current drug information.

FDA APPROVAL INFORMATION

Description / MOA	Colony stimulating factor-1 receptor (CSF-1R)-blocking antibody
Indication Under Review[1]	Treatment of chronic graft-versus-host disease (cGVHD) after failure of at least 2 prior lines of therapy in adults/pediatric patients weighing at least 40kg
Dosage Regimen	0.3 mg/kg (max 35 mg) IV over 30 min. every 3 weeks
Dosage Forms Under Review	Injection 50mg/ml solution in a single dose vial

DISEASE BASICS 101

Chronic Graft versus Host Disease (cGVHD)	**NIH Consensus Criteria for organ scoring**. Score 0-3 (least to most impactful) Grading classified by symptoms, affected organ systems and extent of disease involvement **Mild** ≤ 2 affected organs; no clinically significant functional dysfunction **Moderate** ≥ 3 organs with no dysfunction or ≥ 1 organ with dysfunction but no major disability **Severe** Major disability **Treatment for mild**. Localized therapy (i.e. PUVA, ECP) **Moderate.** Prednisone **Severe.** Prednisone + ruxolitinib

EFFICACY CONSIDERATIONS

Trial	**AGAVE-201**
Design	Phase 2, multinational, randomized trial
Population	Refractory or recurrent cGVHD Active signs/symptoms per NIH consensus criteria s/p ≥ 2 lines of therapy: patients rec'd median 4 lines of therapy Prior therapies included: ibrutinib 23-36%; ruxolitinib 71-79%; belumosudil 20-26% Karnofsky score ≥ 60; Adequate organ function Systemic glucocorticoids, CNI or mTOR inhibitor allowed to continue; other meds to be discontinued
Intervention	Randomized 1:1:1 Axatilimab 0.3mg/kg IV every 2 weeks; Axatilimab 1mg/kg IV every 2 weeks; Axatilimab 3mg/kg IV every 2 weeks
Comparator	None
Results	Primary endpoint: ORR (CR or PR) per NIH Consensus criteria w/in first 6 cycles Secondary endpoint: Reduction in symptoms (i.e. > 5 point reduction in Lee Symptom Scale (LSS) score) **0.3mg-dose group: ORR 74% (95% CI, 63-83)** 1-mg dose group: ORR 67% (95% CI, 55-77) 3-mg dose group: ORR 50% (95% CI, 39-61) Median time to response was less than 2 months **0.3-mg dose group: 60% with > 5 point reduction in LSS score** 1-mg dose group: 69% 3-mg dose group 41% Median time to response was 1.7 months

SAFETY CONSIDERATIONS	
Boxed Warnings	none
Contraindications	none
Other Warnings	Infusion-related reactions. Reported in 18% (Grade 3 or 4: 1.3%); premedicate with antihistamine and antipyretic; monitor for signs and symptoms; slow, interrupt or discontinue rate based on severity Embryo-fetal toxicity. Based on drug mechanism of action, fetal harm may result when given to a pregnant woman; advise effective contraception during and for 30-days after the last dose
Top 5 AEs	Increased AST, infection, increased ALT, decreased phosphate, decreased hgb, viral infection
Drug Interactions	n/a
Safety-specific reports of AGAVE-201	The 0.3mg-dose group had a more favorable toxicity profile. • Any grade periorbital edema occurred in 3% of 0.3mg; 23% of 1mg; 29% of 3mg • Grade 3 or higher AEs in 49% of 0.3mg; 60% of 1mg; 71% of 3mg • Any AE leading to discontinuation: 6% of 0.3mg; 22% of 1mg; 18% of 3mg

PLACE IN THERAPY	DRUG	VANF	CFU	FDA	GUIDELINES /OTHER CONSIDERATIONS
	Axatilimab 0.3mg/kg IV every 2 weeks	TBD	TBD	cGVHD s/p ≥ 2 prior LOT	NCCN v3.2025. After ≥ 2 prior LOT (Cat 2A) • IV formulation • ORR 74% • Responses noted post ibrutinib, ruxolitinib, belumosudil
	Belumosudil 200mg PO daily	NF	yes	cGVHD s/p ≥ 2 prior LOT	NCCN v3.2025. After ≥ 2 prior LOT (Cat 2A) • Oral formulation • ORR 76% in heavily pretreated patients • Responses noted post ibrutinib, ruxolitinib
	Ibrutinib	NF	yes	cGVHD s/p ≥ 1 prior LOT	NCCN v3.2025. After ≥ 1 prior LOT (Cat 2A)
	Ruxolitinib	PA-F	yes	cGVHD s/p ≥ 1 prior LOT	NCCN v3.2025. After ≥ 1 prior LOT (Cat 1)

VHA PLACE IN THERAPY	
Potential Use in VHA	1. Heavily pre-treated patients with cGVHD in the 0.3mg/kg dosing group achieved ORR 75% 2. Organ-specific responses were noted in all organ groups with the 0.3mg/kg dose 3. Responses were noted following prior ibrutinib, prior ruxolitinib and belumosudil (ORR 81, 78 and 75%, respectively) 4. Symptom-reduction was noted in 60% (mLSS > 5-point improvement) 5. Reduction in corticosteroid dose was also notable in 40% of patients by cycle #6 6. Both belumosudil and axatilimab are FDA-approved following at least 2 prior lines of therapy; axatilimab does not appear to have any clear advantage over belumosudil; direct comparative trials have not been performed, but both agents appear to have comparable efficacy 7. Axatilimab has demonstrated responses after progression on ibrutinib, ruxolitinib and belumosudil.

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References

[1] NIKTIMVO (axatilimab-csfr) injection [prescribing information online]. Wilmington, DE. Incyte Corporation. August 2024 Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/761411s000lbl.pdf. Accessed January 2026.

[2] Wolff D, Cutler C, Lee SJ, et al. for the AGAVE-201 Investigators. Axatilimab in Recurrent or Refractory Chronic Graft-Versus-Host Disease. N Engl J Med 2024; 391: 1002-1014.